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Roquefort Therapeutics PLC
11 March 2024

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Roquefort Therapeutics plc

("Roquefort Therapeutics" or the "Company")

Positive Results: Midkine mRNA and STAT-6 siRNA Programs

Pre-clinical development milestones

Roquefort Therapeutics (LSE:ROQ, OTCQB:ROQAF), the Main Market listed biotech company focused on developing first in class medicines in the high value and high growth oncology market, provides an update on the development of the Midkine mRNA and STAT-6 siRNA programs.

Midkine mRNA Program Milestone

Further to the announcement on 6 February 2024, where Roquefort Therapeutics announced that its mRNA therapeutics were undergoing studies in combination with proprietary lipid nanoparticle (LNP) delivery systems, the Company has continued development in validated *in vivo* models. These latest experiments combined the mRNA with a LNP delivery system in a validated *in vivo* model of liver cancer and demonstrated the safety and efficacy in reducing functional Midkine of the novel mRNA LNP combination.

This represents a significant milestone in both the discovery of a novel mRNA therapeutic and in the safe combination with an LNP to allow for the delivery of the mRNA as an anti-cancer medicine. Liver cancer is a leading cause of cancer deaths worldwide, accounting for more than 700,000 deaths each year^[1]. Midkine is associated with liver cancer progression, resistance and prognosis^[2]. A novel therapeutic that targets Midkine expressing liver cancers with the cutting-edge mRNA technology offers the potential for a first-in-class medicine in the \$3B liver cancer market.

STAT-6 siRNA Program Milestone

On 7 August 2023 the Company announced that it had developed four additional siRNA sequences that attack the target STAT-6 (Signal Transducer and Activator of Transcription) and its SH2 (Src-homology-2) domain. The Company has continued the development of these novel STAT-6 medicines in validated *in vitro* models of

colon cancer with the results demonstrating efficacy of the four new siRNA sequences in reducing STAT-6 expression by 40-50%.

Colorectal cancer (CRC) is the third most diagnosed malignancy and a major leading cause of cancer-related deaths worldwide with ~1.9 million new cases per year and accounted for over 900,000 deaths in 2020^[3]. Despite advances in therapeutic regimens, the number of patients presenting with metastatic CRC (mCRC) is increasing due to resistance to therapy^[4]. STAT-6 has been implicated in colorectal cancer development, progression, metastasis, poor survival, and resistance to treatment^[5]. Therefore, a novel therapeutic that targets STAT-6 expressing colon cancers offers the potential for a first-in-class medicine in the \$12B colon cancer market^[6].

Roquefort Therapeutics CEO Ajan Reginald commented:

"We have made significant progress across our pre-clinical drug development programs in Q4 2023 and in Q1 2024. First with the MK cell program announced in February 2024 and today, with the results with our Midkine mRNA and STAT-6 siRNA programs. Both programs highlight Roquefort Therapeutics' ability to select and develop valuable new medicines for the most difficult to treat cancers. Successfully combining our Midkine mRNA with LNP delivery creates potential for a highly promising new medicine for liver cancer and the novel STAT-6 siRNAs have shown great promise in colon cancer.

By focusing on oncology patients with the worst prognosis, who are not well treated with existing medicines, we hope to develop new first-in-class medicines that dramatically improve survival and as a consequence, are the most valuable to potential Pharma acquirers.

Big Pharma is set to face a \$200 billion^[7] fall in revenue from 2024-2030 due to patent expirations while also earning record profits over the last five years. Therefore Big Pharma will have to fill this gap by acquiring new blockbuster medicines to fill this shortfall, and so it is a great time to have our portfolio of potential blockbuster medicines. We remain in active out licencing discussions with Big Pharma companies and a specialist private equity fund across the US, EU and Japan and will update the market should a binding agreement be entered into with one or more partners."

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About Roquefort Therapeutics

Roquefort Therapeutics (LSE:ROQ, OTCQB:ROQAF) is a Main Market listed biotech company developing first in class drugs in the high value and high growth oncology segment prior to partnering or selling to big pharma.

Roquefort Therapeutics' portfolio consists of five novel patent-protected pre-clinical anti-cancer medicines. The highly complementary profile of five best-in-class medicines consists of:

- Midkine antibodies with significant *in vivo* efficacy and toxicology studies;
- Midkine RNA therapeutics with novel anti-cancer gene editing action;
- Midkine mRNA therapeutics with novel anti-cancer approach;
- STAT-6 siRNA therapeutics targeting solid tumours with significant *in vivo* efficacy; and
- MK cell therapy with direct and NK cell-mediated anti-cancer action

For further information on Roquefort Therapeutics, please visit www.roquefortplc.com and @RoquefortTherap on X (formerly Twitter).

[1] American Cancer Society: <https://www.cancer.org/cancer/types/liver-cancer/about/what-is-key-statistics.html>

[2] Filippou, P.S., Karagiannis, G.S. & Constantinidou, A. Midkine (MDK) growth factor: a key player in cancer progression and a promising therapeutic target. *Oncogene* 39, 2040-2054 (2020). <https://doi.org/10.1038/s41388-019-1124-8>

[3] <https://www.wcrf.org/cancer-trends/colorectal-cancer-statistics/>

- [4] Al Bitar S, El-Sabban M, Doughan S, Abou-Kheir W. Molecular mechanisms targeting drug-resistance and metastasis in colorectal cancer: Updates and beyond. *World J Gastroenterol* 2023; 29(9): 1395-1426 [PMID: 36998426 DOI: 10.3748/wjg.v29.i9.1395]
- [5] Ana Catalina Rivera Rugeles, ... Sonia Leon-Cabrera, in [Immunotherapy in Resistant Cancer: From the Lab Bench Work to Its Clinical Perspectives](#), 2021
- [6] <https://www.precedenceresearch.com/colorectal-cancer-therapeutics-market>
- [7] 190 drugs accounting for \$236 billion in sales will lose patent exclusivity by 2030:
<https://www.forbes.com/sites/ohnlamattina/2023/06/26/biopharma-facing-two-major-crisesa-huge-patent-cliff-and-price-controls/>

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Positive Results: Midkine mRNA and STAT-6 siRNA

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Company Announcement - General

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